Commercial PA Criteria

Effective: October 17, 2019

Prior Authorization: Miglustat

Products Affected: miglustat oral capsule, Yargesa capsule

Medication Description: Miglustat functions as a competitive and reversible inhibitor of the enzyme glucosylceramide synthase, the initial enzyme in a series of reactions which results in the synthesis of most glycosphingolipids. The goal of treatment with miglustat is to reduce the rate of glycosphingolipid biosynthesis so that the amount of glycosphingolipid substrate is reduced to a level which allows the residual activity of the deficient glucocerebrosidase enzyme to be more effective (substrate reduction therapy). In vitro and in vivo studies have shown that miglustat can reduce the synthesis of glucosylceramide-based glycosphingolipids.

Covered Uses: Monotherapy for treatment of adult patients with mild to moderate type 1 Gaucher disease for whom enzyme replacement therapy is not a therapeutic option.

Exclusion Criteria: N/A

Required Medical Information:

1. Diagnosis
2. Previous therapy tried/failed

Age Restrictions: 18 years of age and older

Prescriber Restrictions: Prescribed by, or after consultation with, a physician that specializes in the treatment of inherited metabolic disorders

Coverage Duration: 12 months

Other Criteria:

1. Patient has a documented diagnosis of type 1 Gaucher disease; **AND**
2. Documentation that the patient is unable to be treated with enzyme replacement therapy (due to constraints such as allergy, hypersensitivity, or poor venous access).

References:

1. Product Information: ZAVESCA® oral capsules, miglustat oral capsules. Actelion Pharmaceuticals US Inc. (per FDA), South San Francisco, CA, 2014.

Policy Revision history

Rev #	Type of Change	Summary of Change	Sections Affected	Date
1	New Policy	New Policy	All	10/17/2019
2	Update	Added Yargesa	Products affected	12/12/2023